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# **Will the US\$5 million Onasemnogene abeparvosec treatment for spinal muscular atrophy represent ‘value for money’ for the NHS? A rapid inquiry into suggestions that it may be cost-effective.**

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## ABSTRACT

### Objectives

Nusinersen (Spinraza®, Biogen) and onasemnogene abeparvosec (Zolgensma®, Novartis) are novel gene-based therapies for the orphan disease Spinal Muscular Atrophy. Onasemnogene abeparvosec has been allocated an acquisition cost of up to US\$5 million per patient. We undertook a rapid inquiry to evaluate if onasemnogene abeparvosec is likely to be cost effective for the UK NHS.

### Methods

We used publicly available cost effectiveness data and recommended methodology to perform cost utility evaluation of onasemnogene abeparvosec versus best supportive care and nusinersen.

### Results

Our evaluations highlight wide variations in cost and benefit estimates of nusinersen and indicate that onasemnogene abeparvosec is unlikely to represent value for money according to current standards of reimbursement. Results are discussed in the context of reimbursement decisions for orphan diseases.

### Conclusion

Commonly implemented commercial confidentiality practices combined with uncertain data obscure scrutiny and justification of past and present reimbursement decisions for orphan drugs. Future cutting edge expensive therapies will be numerous, they will entail very substantial economic strains. We conclude that there is an urgent and increasing need for the development of improved procedures that can lead to equitable, consistent and transparent decision making.

## INTRODUCTION

The reimbursement of expensive treatments for orphan diseases is contentious for decision makers; many opinions and viewpoints have been expressed. [1-9]

The rare progressive disorder Spinal Muscular Atrophy (SMA) accounts for between 1 in 6,000 to 10,000 births worldwide. About 95% of SMA derives from the 5q mutation. SMA Type 1 is caused by suboptimal-levels of survival motor neurone protein (SMN). Birth prevalence of SMA Type 1 in the USA was estimated at 8.5/100,000 [10]. SMA Type I accounts for approximately 60% of SMA, the age of onset is 2 to 6 months and average survival is a few years. About 100 individuals with SMA are born each year in the UK. SMA is the leading genetic cause of infant death. [11,12] Consensus statement guidelines [13] and recent recommendations [14,15] provide for multidisciplinary standard care. Somewhat controversial very highly dedicated management of care has been undertaken [16,17].

Clinical prospects for SMA Type1 patients recently improved dramatically with the development of two novel gene-based drugs [18,19]: Nusinersen (Spinraza® Biogen) and onasemnogene abeparvosec (Zolgensma® Novartis). Nusinersen delivers copies of an antisense oligonucleotide directed at survival motor neurone gene 2 (SMN 2) via lumbar puncture; this boosts SMN levels from this secondary gene. Intravenously administered Zolgensma® delivers onasemnogene abeparvosec via viral capsids and replaces mutant or missing survival motor neurone gene 1 (SMN1); intra thecal administration is under consideration. Onasemnogene abeparvosec is used in a one-off administration whereas nusinersen requires a series of booster-doses. Novartis acquired onasemnogene abeparvosec with a reportedly US \$8.7 billion purchase of the drug developer AveXis Inc. Novartis have set the one-off acquisition cost at between US\$1.5 million and US\$5 million, reportedly making Zolgensma® the world's most expensive drug [20].

The National Institute for Health and Care Excellence UK (NICE) assessed nusinersen versus best supportive care (BSC) for SMA Type 1 and decided (May 2019) that, as part of a pilot scheme to gather more information, Spinraza® would be offered to "all SMA 1 new-borns" [20] at a commercially confidential drug cost. Recently onasemnogene abeparvosec was approved by the US FDA for the Type I form of SMA, and within the scope of its likely licencing by the European Medicine Agency (EMA) Health Technology Assessment (HTA) agencies (e.g. NICE) have started appraisal processes for its possible use by health care providers. NICE (June 2019) identified BSC and nusinersen as comparators. Novartis and others have intimated that Zolgensma® will be cost-effective [20,21].

## OBJECTIVES

We performed cost utility calculations to evaluate whether, and under what circumstances, multimillion-dollar treatments for SMA Type 1 might represent value for money within the UK NHS.

## DATA SELECTION AND METHODS

We searched for publications comparing the costs and cost-effectiveness of treatments for SMA Type 1: We did not find cost or cost effectiveness data for highly dedicated care [22]. We found 6 potentially relevant publications [21,23-27] relative to best supportive care (BSC), nusinersen and onasemnogene abeparvosec. None compared all three interventions. We identified several Biogen sponsored cost [27] and cost utility studies comparing nusinersen versus BSC using the same Markov model structure [26]; one of these adopted a UK NHS perspective [25] and was included. This model has been critiqued by Canadian [23] and UK HTA agencies [25] and data from these was included for comparative purposes. In addition to these we included a Novartis sponsored US study comparing onasemnogene abeparvosec versus nusinersen [21]. We sourced relevant data from these public domain publications. For the base case (**Error! Reference source not found.**) we extracted the total lifetime cost and QALYs associated with: (i) Spinraza® treatment; (ii) standard care treatment; and (iii) Zolgensma® treatment at different drug acquisition prices from Biogen's submission to NICE [25] and from the Novartis sponsored study of Malone [21]. We adopted a UK NHS perspective and converted values reported in US dollars to UK pounds using 2018 purchasing power parity exchange rates [28]. We combined the data to compare BSC, Zolgensma® and Spinraza®, using the cost utility approach to calculate the incremental cost-effectiveness ratios (ICER) for these comparisons as recommended by Garber and Solomon [29], by Drummond et al., [30], and by Morris et al.,[31].

In bivariate sensitivity calculations we changed QALY yield by  $\pm 20\%$  and, following the approach of Malone et al., lifetime treatment costs were set at 100%, 80%, 60% and 50% of the maximum published values (for Zolgensma® = US\$5 million; for Spinraza® = £4,352,213 (according to Malone) or £2,258,852 (according to Biogen); for best supportive care = £90,299). The resulting pairs of cost and benefit were bootstrapped with 500 iterations [32] and the results depicted in mean centred scatter graphs with 95% confidence intervals [33].

## RESULTS

The values used in our calculations are summarised in Table 1 and the resulting ICER estimates, split by acquisition cost of Zolgensma® and the source of values, are shown in Table 2. For a Zolgensma® list price of \$5million, Spinraza is much more costly and moderately more effective than standard care and it is extendedly dominated by Zolgensma®. The remaining comparison, between Zolgensma® and

best supportive care, results in an ICER over £340k per additional QALY for Zolgensma®. For a Zolgensma® list price of \$2.5million, data from Malone et al., [21] and the Biogen submission [25] suggest that Spinraza® is dominated and extendedly dominated, respectively, and it is disregarded. The ICER for the resulting treatments, Zolgensma® and standard care, is £215k per additional QALY. Thus, in each case, Zolgensma® comes well above commonly used cost-effectiveness thresholds.

Bivariate calculations for the comparison Zolgensma® vs. BSC are summarised in Figure 1 A, again indicating that Zolgensma® is unlikely to be cost effective. It can be argued that the decision by NICE to recommend Spinraza® for newly diagnosed SMA patients suggests that Spinraza® can be regarded as “standard of care” and may well become the only relevant comparator should Zolgensma® be assessed by various HTA agencies. Figure 1B summarises results from bivariate calculations when these two drugs are compared only with each other. These calculated ICERs (£2.8 million / QALY and £15,600 / QALY respectively) highlight the very discordant source data available from Biogen [25] and Malone et al., [21]. The operative cost for Spinraza® within the NHS is commercially confidential as is likely to also be the case for Zolgensma® in the future.

Biogen’s submission to the Canadian HTA agency (CADTH) comparing nusinersen vs. BSC estimated an ICER of Ca\$ 665,570 / QALY (equivalent to £381,195 / QALY) [23]. The CADTH economic review pointed to several shortcomings in the Biogen analysis and independently estimated an ICER of Ca\$ 9.2 million / QALY (~ £5.27 million / QALY). The main reason for the large difference was a much reduced incremental QALY yield relative to that suggested by Biogen (0.25 compared to 4.8); CADTH estimated that a 95% reduction in acquisition cost of nusinersen would be required to bring the ICER to within range of commonly applied norms for cost effectiveness. Biogen’s submission to NICE generated an ICER of £407,605 / QALY [25] similar to that submitted to CADTH. The Biogen sponsored Swedish study [26] reported an ICER of SEK 5.665 million / QALY equivalent to £437,665 / QALY. Canadian and Swedish discount rates for costs and benefits operate at 3% per annum and differ slightly from the 3.5% used in the UK. All these Biogen submitted economic models shared the same structure.

It should be appreciated that Biogen and Malone respectively compared nusinersen vs. BSC and nusinersena vs. onasemnogene abeparvosec so that our calculations are analogous to indirect comparisons.

## DISCUSSION: Reimbursement considerations

Our cost utility calculations use publicly available information and can only be guides where confidential commercial arrangements are operative between decision makers and pharma and where

estimates from potentially rival pharmaceutical companies are discordant. More transparent systems must be preferable. Currently, commercial confidentiality practices mean many interested parties are unable to scrutinise the rationale underpinning many reimbursement decisions [35]. An unintended consequence of greater transparency in pricing is that, in the presence of international reference pricing and parallel trade, lower income countries are likely to be facing higher prices for pharmaceuticals.[36] Nonetheless, it has been argued that carefully designed international reference pricing strategies could offer an effective way towards cost containment.[37]

In the UK from April 2005 the assessment of drugs for rare diseases was undertaken by a National Specialist Commissioning Advisory Group (NSCAG). Burls et al., 2005 [6] pointed out that decisions reached by NSCAG were not accompanied by funding; thus a National body directed services but the costs fell upon primary care trusts responsible for provision to local patients. Primary care trusts operate within a finite budget, monies directed at new expensive treatments represent opportunity costs that necessarily preclude provision of other services. Currently in the UK a rare disease advisory group undertakes “economic evaluation of proposals for services for rare diseases as necessary” [38]. “Plans” are in place for managing orphan drugs should a no-deal Brexit materialise [5]. Eculizumab (Soliris®, Alexion), previously deemed the world’s most expensive drug, was directly adopted by UK ministerial decree for the treatment of paroxysmal nocturnal haemoglobinuria, thereby bypassing or overarching decision at other levels [39,40]. Whether treatments for orphan diseases are, have been, or should be, considered according to different criteria to those for other treatments is contentious [1,3,4,41]. Be that as it may, to divorce the reimbursement decision about expensive orphan drugs from the payers who must ultimately budget for their provision does not seem sensible.

Although inherently simple, we believe our calculations are reliable in the context of the information available from the publicly available data. Biogen[20] [26] and Malone et al., 2019 [21] employ complex modelling to relate the observed trajectory of muscular development, to overall survival and quality of life. Striking differences result in their estimation of QALYs delivered by Spinraza® (5.29 versus 7.86) and in estimating the lifetime cost of Spinraza® (£4.35 million versus £2.26 million). The relationship between improved motor function, survival and quality of life is very uncertain and potential health improvements beyond a few years are completely undocumented. Deaths among Zolgensma® treated patients have been observed and are / have been investigated to determine if they may be treatment related [42]. Combination of gene therapies with non-invasive ventilation plus mechanical airway clearance [22] might improve outcomes. Further clinical investigations are necessary.

## CONCLUSION

With the successful recent development of “gene-based therapies” there is now very substantial innovative impetus for effective treatments for rare genetic diseases [43] and this will include expensive gene-based approaches for neurological and other conditions. The economic implications are substantial and need to be openly addressed. Our results highlight deficiencies that currently can influence reimbursement decisions that may profoundly affect clinical prospects for individuals with orphan diseases. Future decisions need to be more transparent, equitable and consistent, making desirable a mechanism for ring-fencing of national funds for orphan diseases.



#### ARTICLE HIGHLIGHTS BOX

- onasemnogene abeparvosec and nusinersen are expensive new gene-based therapies for the orphan disease SMA1, they represent harbingers for development of gene therapies for many rare diseases in the near future.
- The treatment cost of these therapies is in the multiple million £ range per individual patient.
- Utilising data from industry sponsored cost utility analyses we evaluated their cost effectiveness from the perspective of the UK NHS.
- Our evaluations indicate that these therapies are unlikely to represent value for money for the NHS but that accurate assessment is difficult due to discrepant estimates from different industry sources and the commercially confidential cost arrangements established between industry and decision makers.
- Reimbursement decisions for orphan drugs need to be equitable, consistent and transparent; improved policies are required.

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Table 1. Total Cost and QALY values used in calculations

Values of total cost and QALY used in calculations		
Treatment and parameter	Value used	Source
<b>Nusinersen (Spinraza®)<sup>Δ</sup></b>		
Total lifetime costs (Table 8 Malone et al. 2019)	£4,352,213	[21]
Total lifetime costs (from Biogen submission to NICE)	£2,258,852	[25]
Total lifetime QALYs (Table 8 Malone et al. 2019)	5.29	[21]
Total lifetime QALYs (from Biogen submission to NICE )	7.86	[25]
<b>Onasemnogene abeparvosec (Zolgensma®)</b>		
Total lifetime costs (for a \$2.5million acquisition price)	£2,903,706	[21]
Total lifetime costs (for a \$5million acquisition price)	£4,576,047	[21]
Total lifetime QALYs (Table 8 Malone et al. 2019)	15.65	[21]
<b>Best supportive care</b>		
Total lifetime costs	£26,637	[25]
Total lifetime QALYs	2.58	[25]

<sup>Δ</sup> Biogen's Spinraza data is likely for the 5q mutant form of the disease, the target population for Spinraza.

Malone et al., published data for Spinraza and Zolgensma is based on natural history evidence and it is unclear if this is specific for the 5q form of the disease. The carrier frequency of 5q has been estimated by Verhaart et al., 2017 [34]

Table 2. Calculated incremental cost-effectiveness ratios (ICERs)

<b>Incremental Cost Effectiveness Ratios (ICERs) split according to source data and Zolgensma® acquisition cost</b>			
<b>Acquisition cost of Zolgensma® US\$5 million</b>			
Nusinersen (Spinraza®) data from Malone et al.		Nusinersen (Spinraza®) data from Biogen	
Treatment	ICER	Treatment	ICER
Best supportive care	-	Best supportive care	-
Spinraza®	Extendedly dominated	Spinraza®	Extendedly dominated
Zolgensma®	£343, 209	Zolgensma®	£343,209
<b>Acquisition cost of Zolgensma® US\$2.5 million</b>			
Nusinersen (Spinraza®) data from Malone et al.		Nusinersen (Spinraza®) data from Biogen	
Treatment	ICER	Treatment	ICER
Best supportive care	-	Best supportive care	-
Zolgensma®	£215,257	Spinraza®	Extendedly dominated
Spinraza®	Dominated	Zolgensma®	£215,257

